## P0195-GB01

## **CLAIMS:**

- 1. The compound 3,4,5-piperidinetriol, 2-(hydroxymethyl)-1-[(4-(pentyloxy)phenyl)methyl]-, (2S,3S,4R,5S) or a pharmaceutically acceptable salt or prodrug thereof.
- 2. A compound as defined in claim 1 for use in medicine.
- 3. A pharmaceutical composition comprising a compound, as defined in claim 1, together with one or more pharmaceutically acceptable carriers, excipients and/or diluents.
- 4. A process for the preparation of a compound, as defined in claim 1, which comprises:
- a) reacting a compound of formula (II):

with 4-(pentyloxy)benzaldehyde using NaBH<sub>3</sub>CN or a supported reagent in acetic acid-methanol or HCl-methanol, or using NaBH(OAc)<sub>3</sub> in a solvent, or

b) deprotection of a compound of formula (III):

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wherein P, which may be the same or different, are hydroxy protecting groups.

- 5. The use of a compound, as defined in claim 1, in the manufacture of an inhibitor of glucosylceramide synthase.
- 6. The use of a compound, as defined in claim 1, in the manufacture of a medicament for the treatment of a glycolipid storage disease.
- 7. The use as claimed in claim 6, wherein the glycolipid storage disease is Gaucher disease, Sandhoff's disease, Tay-Sachs disease, Fabry disease or GM1 gangliosidosis.
- 8. The use of a compound, as defined in claim 1, in the manufacture of a medicament for the treatment of Niemann-Pick disease type C, mucopolysaccharidosis type I, mucopolysaccharidosis type IIIA, mucopolysaccharidosis type IIIB, mucopolysaccharidosis type VI or mucopolysaccharidosis type VII,  $\alpha$ -mannosidosis or mucolipidosis type IV.
- 9. The use of a compound, as defined in claim 1, in the manufacture of a medicament for the treatment of cancer in which glycolipid synthesis is abnormal.

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- 10. The use according to claim 9, wherein the cancer in which glycolipid synthesis is abnormal is selected from brain cancer, neuronal cancer, neuroblastoma, renal adenocarcinoma, malignant melanoma, multiple myeloma or multi-drug resistant cancers.
- 11. The use of a compound, as defined in claim 1, in the manufacture of a medicament for use in the treatment of Alzheimer's disease, epilepsy, stroke, Parkinson's disease or spinal injury.
- 12. The use of a compound, as defined in claim 1, in the manufacture of a medicament for use in the treatment of diseases caused by infectious microorganisms which utilize glycolipids on the surface of cells as receptors for either the organism itself or for toxins produced by the organism, or infectious microorganisms for which the synthesis of glucosylceramide is an essential or important process.
- 13. The use of a compound, as defined in claim 1, in the manufacture of a medicament for use in the treatment of diseases associated with abnormal glycolipid synthesis.
- 14. The use according to claim 13, wherein the diseases associated with abnormal glycolipid synthesis are selected from polycystic kidney disease, diabetic renal hypertrophy or atherosclerosis.
- 15. The use of a compound, as defined in claim 1, in the manufacture of a medicament for the treatment of a condition treatable by the administration of a ganglioside such as GM1 ganglioside.
- 16. The use of a compound, as defined in claim 1, in the manufacture of a medicament for use in reversibly rendering a male mammal infertile.
- 17. The use of a compound, as defined in claim 1, in the manufacture of a medicament for the treatment of obesity.
- 18. The use of a compound, as defined in claim 1, in the manufacture of a medicament for the treatment of inflammatory diseases or disorders associated with macrophage recruitment and activation.
- 19. The use of a compound according to claim 18, wherein the inflammatory disease or disorder associated with macrophage recruitment and activation is selected from rheumatoid arthritis, Crohn's disease, asthma or sepsis.
- 20. A compound of formula (III):

wherein P, which may be the same or different, are hydroxy protecting groups.

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